

# Lambert-Eaton Myasthenic Syndrome (LEMS) Registry (LEMS-01)

**First published:** 21/07/2014

**Last updated:** 29/01/2021

Study

Finalised

## Administrative details

### EU PAS number

EUPAS6106

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### Study ID

39278

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### DARWIN EU® study

No

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### Study countries

-  France
  -  Germany
  -  Italy
  -  Spain
  -  United Kingdom
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## **Study description**

Lambert-Eaton Myasthenic Syndrome (LEMS) is a rare autoimmune disorder that affects voltage-gated calcium channels on the pre-synaptic membrane of the nerve-muscle (neuromuscular) junction. LEMS is estimated to affect 1 in 100,000 people in the European Community and is clinically characterized by weakness and frequent fatigue (mainly of the legs and trunk), and is associated with autonomic dysfunction (e.g. impotence, dry mouth, constipation). The onset of symptoms is usually gradual and insidious. The age at onset is typically  $\geq 40$  years in patients with cancer and between 20 and 50 years in those without. Slightly more men than women are affected. LEMS has also been reported in children, sometimes associated with neuroblastoma, but is an extremely rare condition in this patient group. In January 2010 the EU approved the Marketing Authorisation of 3, 4-DAP Phosphate (Amifamdripine, Firdapse®) for the treatment of LEMS. Firdapse is the only approved 3, 4-DAP compound for the treatment of LEMS. The purpose of the LEMS registry is to collect additional data on the long term safety and efficacy of Firdapse for patients who have been prescribed Firdapse by their treating physician. This registry will also increase knowledge about the course of disease in patients with LEMS by evaluating neurological and muscular function. The LEMS registry is a voluntary multi-centre, multinational, observational program for patients with LEMS disease, intended to track the routine clinical outcomes of patients with LEMS over time. As per condition of the EU Marketing Authorisation, the registry will also track the use of treatment for LEMS including drugs other than Firdapse. The data collected by the registry are intended to enable better characterisation of the natural history of LEMS. All patients with a confirmed diagnosis of LEMS disease may be eligible to participate in this program. No experimental treatments or assessments are involved in this program.

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## **Study status**

Finalised

## **Research institutions and networks**

## Institutions

### BioMarin Pharmaceuticals

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

Multiple centres: 30 centres are involved in the study

## Contact details

### Study institution contact

Program Director [medinfoeu@BMRN.COM](mailto:medinfoeu@BMRN.COM)

Study contact

[medinfoeu@BMRN.COM](mailto:medinfoeu@BMRN.COM)

### Primary lead investigator

Program Director

Primary lead investigator

## Study timelines

**Date when funding contract was signed**

Planned: 01/10/2009

Actual: 09/01/2014

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**Study start date**

Planned: 31/03/2010

Actual: 25/05/2010

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**Date of final study report**

Planned: 28/06/2020

Actual: 21/02/2020

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

BioMarin Clinical Limited

## Regulatory

**Was the study required by a regulatory body?**

Yes

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**Is the study required by a Risk Management Plan (RMP)?**

EU RMP category 3 (required)

## Methodological aspects

### Study type

### Study type list

**Study topic:**

Human medicinal product

Disease /health condition

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**Study type:**

Non-interventional study

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**Scope of the study:**

Disease epidemiology

Effectiveness study (incl. comparative)

**Data collection methods:**

Combined primary data collection and secondary use of data

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**Main study objective:**

To obtain observational safety data (identification, frequency and severity) on patients diagnosed with LEMS. To monitor for safety signals in patients treated with Firdapse including long-term treatment. To evaluate the outcome of pregnancies in patients treated with Firdapse. To gather the same observational information from patients with LEMS not treated with Firdapse...

## Study Design

**Non-interventional study design**

Other

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**Non-interventional study design, other**

Observational

## Study drug and medical condition

**Medicinal product name**

FIRDAPSE

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**Medical condition to be studied**

Myasthenic syndrome

## Population studied

**Short description of the study population**

Patients diagnosed with Lambert-Eaton Myasthenic Syndrome (LEMS).

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**Age groups**

- Adults (18 to < 46 years)
  - Adults (46 to < 65 years)
  - Adults (65 to < 75 years)
  - Adults (75 to < 85 years)
  - Adults (85 years and over)
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**Special population of interest**

Other

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**Special population of interest, other**

Lambert-Eaton Myasthenic Syndrome patients

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**Estimated number of subjects**

105

## Study design details

## Data analysis plan

Registry data will be analyzed as per the program's (SAP) and will be reported periodically to the European Medicines Agency (EMA) as per post marketing commitment. Longitudinal prospective and retrospective data may be collected. Demographic and baseline characteristics will be summarized. Frequencies will be presented for the categorical variables (e.g. sex and ethnicity), and descriptive statistics will be presented for continuous variables (e.g. height, weight, and age). Periodical descriptive statistical reports and final statistical analysis may also include: Patient accrual and follow-up Exposure to Firdapse (dose, duration) Clinical response Pregnancy outcomes as appropriate Adverse Events (AE) including Serious AEs. AEs will be recorded as available.

## Data management

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

### Data sources (types)

[Disease registry](#)

[Electronic healthcare records \(EHR\)](#)

[Other](#)

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## **Data sources (types), other**

Prospective patient-based data collection, Prescription event monitoring

## Use of a Common Data Model (CDM)

### **CDM mapping**

No

## Data quality specifications

### **Check conformance**

Unknown

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No